
Genes at the heart of heart deformities found through stem cell studies

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CIRM grantees at The Gladstone Institutes have, over the past few years, been hard at work learning about the origins of heart deformities by studying how stem cells mature into heart tissue.

What they've learned is that small relatives of DNA, called micro-RNAs, help control when and how cells mature into heart tissue (blogged about here and here) or blood vessels (blogged about here). In recent work out of the lab of Deepak Srivastava, they also discovered three genes are activated by a key micro-RNA whose absence can lead to heart deformities. That work is published in the April 17 *Developmental Cell*.

The work was in fruit flies, but many basic discoveries in fruit flies directly translate to humans.

Isabelle King, who works with Srivastava at the Gladstone Institutes and led the study, said discovering those genes could help scientists understand and treat cases where the heart failed to form properly in development. A press release from Gladstone quotes King:

“In the fetal heart, subtle changes in gene dosage and timing can yield heart defects in children.”

This work is a great example of how basic stem cell research can lead to new areas to explore for disease therapies. People often think of stem cell therapies as exclusively transplantation therapies in which stem cells and their derivatives are transplanted into a diseased organ to restore function. We do fund scientists trying to do just that, but we also fund basic stem cell scientists who are discovering how diseases arise, and the genes responsible. These discoveries made possible by studying stem cells could lead to new drugs or other interventions that have nothing to do with transplantation.

On the topic of basic research, we'll be funding our third round of Basic Biology Awards at our governing board meeting next week (here are review summaries of those applications). These awards are intended to foster the kinds of basic stem cell and disease discoveries that keep new ideas - and eventual cures - flowing.

Developmental Cell, April 17, 2011

CIRM funding: Deepak Srivastava (RC1-00142); Li Quan (TG2-01160)

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